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Compassionate Drug Use

What is compassionate drug use?

Compassionate drug use means making a new, unapproved drug available to treat a seriously ill patient when no other treatments are available.

Drugs that are being tested but have not yet been approved by the US Food and Drug Administration (FDA) are called **investigational drugs**. These drugs are normally available **only** to people who are taking part in a [clinical trial](#)¹.

Being able to use one of these drugs when you are not in a clinical trial is most commonly referred to as compassionate drug use.

Is compassionate drug use legal?

Compassionate drug use is legal, but it's limited to people who meet certain conditions.

Who might benefit from compassionate drug use?

Patients with serious or life-threatening conditions who can't get treatment with an unapproved drug through a clinical trial might benefit from compassionate use, if it's available.

How can people access compassionate drug use?

The usual way to get treatment with an unapproved drug is through a clinical trial. But many people with life-threatening diseases can't find clinical trials that they can be a part of. This could be because they live too far from locations where the studies are being done, or because they don't meet the requirements.

People who aren't in clinical trials might be able to get access to an unapproved drug from the company that makes it in 2 ways:

- Through **expanded access programs (EAPs)**.
- Through **Right to Try**.

Here are some important things to know about drugs that may be available through compassionate use:

- Not all drugs are available for compassionate use, and drug companies aren't required to supply their drugs through compassionate use. Each drug company has different policies and processes, but laws require all drug company to publicly post contact information for compassionate use requests.
- There may be very limited amounts of a drug available for compassionate use, or drug companies might only have enough drug for use in clinical trials. Some drug companies will supply a drug through compassionate use for free, but others might charge patients. Most insurance companies will not pay for the costs of the investigational drugs themselves, even with compassionate use. There might also be other costs, such as the clinic's charges for giving the drug and monitoring your response; those might not be covered by health insurance.

Expanded access programs (EAPs)

The FDA accepts applications for expanded access every day of the week. It usually takes 4 days to process a non-emergency request, and less than one day to process an emergency request. Requests are made through your doctor. The FDA approves most of these requests, but often requires changes to the study protocol for the individual to assure safety. These may be changes in dose of a drug or changes to safety monitoring.

In general, EAPs are for patients who meet all of these conditions:

- Have a serious and life-threatening condition
- Are not eligible for any current clinical trial that's using the drug
- Have no other comparable treatment options
- Are likely to have benefits that outweigh the risks involved

The Right to Try Act

A federal law passed in 2018 gave patients another path to access unapproved drugs, without needing the approval of the FDA. This pathway is commonly referred to as Right to Try. Right to Try laws do not replace EAPs but provide another way to access unapproved drugs.

It's important to understand that Right to Try does not actually give patients the right to try any unapproved drug they wish to try. Instead, it gives them the right to request access to an unapproved drug from the company that makes it, without having to go through the FDA.

Bypassing the FDA does not necessarily mean that such access will be granted. There are a couple of reasons access may be denied, including:

- If the patient does not meet the criteria spelled out in the law
- If the drug company refuses to provide the drug, or they aren't able to make enough of it for all of the people requesting it

Bypassing the FDA also means there may be less guidance for how the drug is being given.

To be eligible for Right to Try, a person must:

- Be diagnosed with a life-threatening disease or condition
- Have tried all approved treatment options for the disease or condition
- Have a doctor certify that they are unable to participate in a clinical trial for the investigational drug
- Give written informed consent that they understand the risks of taking the investigational drug

In addition, the drug itself must have already been through a [phase I clinical trial](#)². (This is the earliest phase of clinical trials, which is generally intended to start looking at the safety of the drug and the proper dose to use.)

To learn more, see [the FDA's information about](#)³[Right to Try](#).⁴

How are EAPs and Right to Try different? How are they the same?

There are some differences between EAP and Right to Try pathways. Some of these

might be good or bad, depending on the situation.

	EAPs	Right to Try
Specific criteria are required for patients to get access to a drug being studied, such as: <ul style="list-style-type: none"> • The patient must have a life-threatening condition • The drug must be being tested in clinical trials • The patient isn't eligible for clinical trials of the drug 	Yes	Yes
FDA approval for access to a drug being studied is required.	Yes	No
Request for drug access must be made by a licensed doctor.	Yes	Yes
Includes some oversight about drug safety by the FDA and the treatment center's institutional review board (IRB)	Yes	No
Drug and clinical trial sponsors can decline requests for various reasons.	Yes	Yes
If access is approved, insurance companies are required to pay the cost of drug and associated administration/treatment.	No	No
Cost charged to a patient is limited to direct drug costs, although sometimes indirect charges to the patient are possible.	Yes	Yes
Adverse events must be immediately reported to FDA.	Yes	No

What should I ask my doctor about compassionate drug use?

Here are some questions you might want to ask if you and your doctor are discussing compassionate drug use:

- Are there any approved treatments that I haven't tried?
- Is there any evidence to support the use of this drug to treat my type of cancer?
- What makes you think this drug could help me?

- In what way do you think this drug is likely to work better than an approved drug?
- What are the known risks and benefits of treatment with this drug?
- Will the drug company give me the drug for free? If not, how is it to be paid for? What costs will I have to pay to get the drug? Will my insurance cover any costs?
- Should we consider expanded access (EAP) or a Right to Try path? What are the pros and cons of each?
- What will we have to do to get access to this drug?
- How long do you think it will take for me to get access to this drug?

Applying for compassionate use through an expanded access program (EAP)

Your doctor or one of the office staff will work with you on this process. If you and your doctor find a drug you want to get access to through an EAP, your doctor will need to contact the drug company to see if they would be willing to provide the drug as part of your treatment. If so, the next step is to contact the FDA's Center for Drug Evaluation and Research (CDER) at 1-301-796-3400 or fda.gov/drugs⁵ or the after-hours FDA Emergency Call Center at 1-866-300-4374 (toll free). See the [FDA's information on EAPs](#)⁶ to learn more.

Applying for compassionate use through Right to Try

For this pathway, your doctor will first need to contact the drug company to find out if you might be able to access the drug. (As a patient, you might be able to make the initial contact with the company to find out about your eligibility or if the drug is available, but the request for the drug will need to come from your doctor.) Your doctor or one of the office staff will work with you on this process. Then, your doctor will need to follow certain steps and work with you to get access if the drug is available.

Hyperlinks

1. www.cancer.org/treatment/treatments-and-side-effects/clinical-trials.html
2. www.cancer.org/treatment/treatments-and-side-effects/clinical-trials/what-you-need-to-know/phases-of-clinical-trials.html
3. www.fda.gov/patients/learn-about-expanded-access-and-other-treatment-options/right-try
4. www.fda.gov/patients/learn-about-expanded-access-and-other-treatment-options/right-try

5. www.fda.gov/drugs/drug-information-consumers/find-information-about-drug
6. www.fda.gov/news-events/public-health-focus/expanded-access

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